

## Leukemia Fact Sheet

CIRM funds many projects seeking to better understand leukemia and to translate those discoveries into new therapies.

### Description

Leukemia is a cancer of the blood cells. Although leukemia is among the most common childhood cancers, it most often occurs in older adults and is slightly more common in men than women. In 2016 there were an estimated 60,000 new cases with around 24,000 people dying of the disease.

The stem cells in the bone marrow normally form all cells of the blood system, including the red blood cells, platelets, and immune cells. In people with leukemia, the bone marrow stem cells create abnormal immune cells that aren't able to carry out their normal job of fighting infection.

Eventually, these abnormal cells crowd out the normal blood cells. Without a sufficient population of working blood cells, people with leukemia develop symptoms such as anemia, bleeding and infections.

Recent research has shown that in addition to these abnormal white cells, leukemia patients also have a small population of cells called leukemia stem cells. Scientists suspect that these cells evade treatments that kill leukemia cells and then later go on to cause a relapse. The goal of stem cell research for leukemia is to find ways of destroying these leukemia stem cells.

**Catriona Jamieson of the UC San Diego Moores Cancer Center discusses a clinical trial for a pre-leukemia condition that was based in part on CIRM funding**

### Clinical Stage Programs

#### University of California, San Diego

A team at UC San Diego is testing the safety of a monoclonal antibody called cirmtuzumab that targets cancer stem cells in a Phase 1

clinical trial. It's named after CIRM because we helped fund the research that led to its development. Cirmtuzumab is designed to attach to a protein, called ROR1, that is found on the surface of chronic lymphocytic leukemia (CLL) cells but is rarely found on healthy cells. The team hopes cirmtuzumab will target the cancer cells, blocking their ability to grow and/or survive.

- Read a summary of this project
- Learn more about this clinical trial

The same UCSD team is also testing cirmtuzumab in combination with an approved cancer fighting drug call Ibrutinib, to target cancer stem cells in a separate Phase 1 trial. The hope is that combining Cirmtuzumab with Ibrutinib will improve cancer remission and long-term cancer control in patients.

- Read a summary of this project
- Learn more about this clinical trial

## CIRM Grants Targeting Leukemia





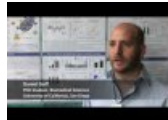
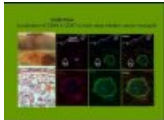




Researcher name	Institution	Grant Title	Grant Type	Approved funds	
Irving Weissman	Stanford University	Identification and isolation of transplantable human hematopoietic stem cells from pluripotent cell lines; two steps from primitive hematopoiesis to transplantable definitive cells, and non-toxic conditioning of hosts for hematopoietic stem cell transplan	Tools and Technologies III	\$1,271,952	
Jacob Corn	University of California, Berkeley	Genome editing for causation and reversion of MPN-associated mutations in human hematopoietic stem cells	Inception - Discovery Stage Research Projects	\$235,800	
Dan Kaufman	University of California, San Diego	Targeted off-the-shelf immunotherapy to treat refractory cancers	Quest - Discovery Stage Research Projects	\$1,936,936	
Jagath Junutula	Cellerant Therapeutics	Development of CLT030-ADC, a Leukemic Stem Cell Targeting Antibody-Drug-Conjugate, for Treatment of Acute Myeloid Leukemia	Late Stage Preclinical Projects	\$6,863,755	
Markus Muschen	Children's Hospital of Los Angeles	Dual targeting of tyrosine kinase and BCL6 signaling for leukemia stem cell eradication	Early Translational II	\$850,769	
Michael Pulsipher	Children's Hospital of Los Angeles	Antiviral Cellular Therapy for Enhancing T-cell Reconstitution Before or After Hematopoietic Stem Cell Transplantation (ACES)	Clinical Trial Stage Projects	\$4,825,587	
Catriona Jamieson	University of California, San Diego	Derivation and Characterization of Cancer Stem Cells from Human ES Cells	SEED Grant	\$616,305	
Matthew Spear	Poseida Therapeutics	Clinical Study of T stem cell memory (Tscm)-based CAR-T cells in Patients with Multiple Myeloma	Clinical Trial Stage Projects	\$19,813,407	

Catriona Jamieson	University of California, San Diego	Preclinical development of a pan Bcl2 inhibitor for cancer stem cell directed therapy	Early Translational II	\$3,103,041	
John Zaia	City of Hope	The Innovation-Alpha Clinic for Cellular Therapies (I-ACT) – A Program for the Development and Delivery of Innovative Cell-based Treatments and Cures for Life-threatening Diseases.	Alpha Stem Cell Clinics	\$8,000,000	
Catriona Jamieson	University of California, San Diego	A Splicing Modulator Targeting Cancer Stem Cells in Acute Myeloid Leukemia	Therapeutic Translational Research Projects	\$2,700,420	
Markus Muschen	University of California, San Francisco	Dual targeting of tyrosine kinase and BCL6 signaling for leukemia stem cell eradication	Early Translational II	\$2,756,536	
David Baylink		Bone Marrow Targeting of Hematopoietic Stem Cells Engineered to Overexpress 25-OH-VD3 1- $\alpha$ -hydroxylase for Acute Myeloid Leukemia Therapy	Inception - Discovery Stage Research Projects	\$178,967	
Dennis Carson	University of California, San Diego	Development of Highly Active Anti-Leukemia Stem Cell Therapy (HALT)	Disease Team Research I	\$19,999,826	
Mehrdad Abedi	University of California, Davis	Stem Cell Gene Therapy for HIV Mediated by Lentivector Transduced, Pre-selected CD34+ Cells in AIDS lymphoma patients	Clinical Trial Stage Projects	\$8,414,265	
Irving Weissman	Stanford University	Development of Therapeutic Antibodies Targeting Human Acute Myeloid Leukemia Stem Cells	Disease Team Research I	\$18,759,276	
Catriona Jamieson	University of California, San Diego	Derivation and Characterization of Myeloproliferative Disorder Stem Cells from Human ES Cells	New Faculty II	\$3,065,572	
Emmanuelle Passegue	University of California, San Francisco	Mechanisms Underlying the Responses of Normal and Cancer Stem Cells to Environmental and Therapeutic Insults	New Faculty II	\$2,124,488	
John Chute	University of California, Los Angeles	Protein tyrosine phosphatase - sigma inhibitors for hematopoietic regeneration	Quest - Discovery Stage Research Projects	\$2,116,708	
Hanna Mikkola	University of California, Los Angeles	Mechanisms of Hematopoietic stem cell Specification and Self-Renewal	New Faculty I	\$2,286,900	
Paul Finnegan	Angiocrine Bioscience, Inc.	Development of AB-110: genetically-modified endothelial cells plus expanded cord blood hematopoietic stem cells as a transplantation therapy	Late Stage Preclinical Projects	\$3,797,117	

Tannishtha Reya	University of California, San Diego	Reprogramming human stem cells for blood cell generation	Inception - Discovery Stage Research Projects	\$210,060	
Chong-Xian Pan	University of California, Davis	Combinatorial Chemistry Approaches to Develop Ligands against Leukemia Stem Cells	New Faculty I	\$2,386,409	
Markus Müschen	City of Hope	Lgr5-mediated self-renewal in B cell selection and leukemia-initiation	Quest - Discovery Stage Research Projects	\$2,186,520	
Gay Crooks	University of California, Los Angeles	Forming the Hematopoietic Niche from Human Pluripotent Stem Cells	Basic Biology III	\$1,252,857	
Mehrdad Abedi	University of California, Davis	Stem Cell Gene Therapy for HIV in AIDS Lymphoma Patients	Disease Team Therapy Planning I	\$66,880	
Paul Finnegan	Angiocrine Bioscience, Inc.	AB-110-001 Phase 1b Trial and Related Activities to Support Clinical Development of AB-110	Clinical Trial Stage Projects	\$5,000,000	
Michael Cleary	Stanford University	Prostaglandin pathway regulation of self-renewal in hematopoietic and leukemia stem cells	Basic Biology IV	\$1,244,455	
Thomas Kipps	University of California, San Diego	A Phase 1b/2a Study of the ROR1-Targeting Monoclonal Antibody, Cirmtuzumab, and the Bruton Tyrosine Kinase Inhibitor, Ibrutinib, in B-Cell Cancers	Clinical Trial Stage Projects	\$18,292,674	
Ann Zovein	University of California, San Francisco	Human endothelial reprogramming for hematopoietic stem cell therapy.	New Faculty Physician Scientist	\$2,319,784	
Colleen Delaney	Nohla Therapeutics Inc	A Phase 2 Open-Label, Multi-Center, Randomized, Controlled, Optimal Dose-Finding Study of DCC-UCB in Adults Receiving High Dose Chemotherapy for AML	Clinical Trial Stage Projects	\$6,922,109	
Thomas Kipps	University of California, San Diego	Therapeutic Eradication of Cancer Stem Cells with UC-g61 (Cirmtuzumab)	Disease Team Therapy Development III	\$4,179,598	
Mark Chao	Forty Seven Inc.	A Phase 1b Trial of Hu5F9-G4 Monotherapy or Hu5F9-G4 in Combination with Azacitidine in Patients with Acute Myeloid Leukemia	Clinical Trial Stage Projects	\$5,000,000	

Irving Weissman	Stanford University	Clinical Investigation of a Humanized Anti-CD47 Antibody in Targeting Cancer Stem Cells in Hematologic Malignancies and Solid Tumors	Disease Team Therapy Development III	\$6,505,568	
Mark Walters	Children's Hospital of Oakland Research Institute	University of California, San Francisco (UCSF) CIRM Alpha Stem Cell Clinic	Alpha Stem Cell Clinics Network Expansion	\$7,999,999	
					Total: \$177,284,540.00

## CIRM Leukemia Videos

 <p><b>Stem Cell Therapies for Leukemia: Marching Toward the Clinic</b></p>	 <p><b>Anica Sayoc, City of Hope - CIRM Stem Cell #SciencePitch</b></p>	 <p><b>Catriona Jamieson, UCSD - CIRM Stem Cell #SciencePitch</b></p>	 <p><b>Irving Weissman, Stanford - CIRM Stem Cell #SciencePitch</b></p>
 <p><b>Leukemia: Advancing Stem Cell Therapies - 2011 CIRM Grantee Meeting</b></p>	 <p><b>Spotlight on Basic Research: Irv Weissman</b></p>	 <p><b>Spotlight on Leukemia: Welcoming Remarks</b></p>	 <p><b>Spotlight on Leukemia: Catriona Jamieson, M.D.</b></p>
 <p><b>Spotlight on Leukemia: Clinical Trial Participants</b></p>	 <p><b>Progress and Promise in Leukemia</b></p>		

## News and Information

- CIRM Stem Cellar blogs on blood cancer
- Leukemia under the microscope (San Diego Union Tribune)
- Bad Seeds: Cancer's Ultimate Source (Stanford Medicine)
- The True Seeds of Cancer (Stanford Medicine)
- From Bench to Bedside in a Year (UC San Diego)
- Living with Leukemia: Theresa Blanda (CIRM)

## Resources

- NIH: Leukemia information
- Find a clinical trial near you: NIH Clinical Trials database

- Leukemia and Lymphoma Society
- Leukemia Research Foundation
- Stem Cell Network blood cancers page
- Family Caregiver Alliance
- National Family Caregivers Association

**Find Out More:**

[Stem Cell FAQ](#) | [Stem Cell Videos](#) | [What We Fund](#)

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**Source URL:** <https://www.cirm.ca.gov/our-progress/disease-information/leukemia-fact-sheet>